

# The Early Access to Medicines Scheme

On 7 April 2014 the Medicines and Healthcare products Regulatory Agency (MHRA) launched the Early Access to Medicines Scheme, with the aim of providing patients in the UK who have life-threatening or seriously debilitating conditions access to medicines that do not yet have marketing authorization and which address an unmet medical need. The scheme could potentially allow doctors and their patients access to innovative medicines up to 1 year earlier than at present.

Overall the scheme has been well received by patient groups, medical research charities, clinical researchers and industry (Association of the British Pharmaceutical Industry and BioIndustry Association, 2014). In order for the scheme to be a success, it is vital that there is now engagement at all stages – from the initial application to clinical uptake.

## Early Access to Medicines Scheme in practice

Early Access to Medicines Scheme is a three-step voluntary evaluation process. It is intended to allow patients access to therapies that have completed phase III trials, but may be applied to those that have completed phase II trials in exceptional circumstances.

Step I is based around a promising innovative medicine designation, which is similar to the Food and Drug Administration's 'Breakthrough Therapies Designation' (Food and Drug Administration, 2014). The MHRA will approve or decline the promising innovative medicine designation based on both non-clinical and clinical data provided in the promising innovative medicine application. The promising innovative medicine designation can be based on phase I/II data and the MHRA will not make positive or negative promising innovative medicine designations publicly available.

Once a positive promising innovative medicine designation is obtained, applicants can then proceed to step II, the Early Access to Medicines Scheme scientific opinion. This scientific opinion will

describe the benefits and risks of the medicine and is based on information submitted to the MHRA by the applicant. The scientific opinion lasts for up to 1 year, and the MHRA must be provided with regular updates on the performance of the drug during this period. Positive opinions will be made publicly available in a public assessment report on the MHRA's website.

The public assessment report will include a description of the product, information on the key clinical studies, and will highlight any uncertainties with the treatment and any measures put in place to manage these risks. It is anticipated that the public assessment report will be used to support clinicians and patients in making a treatment decision on the use of the medicine before its licence has been approved. National Institute for Health and Care Excellence are also keen to discuss how some of the information they produce, such as the evidence summary of off-label or unlicensed medicines, could be of use in communicating an Early Access to Medicines Scheme opinion to doctors, patients and the wider NHS.

The promising innovative medicine and scientific opinion application documents can be accessed via the MHRA website (MHRA, 2014).

Step III is commissioning of the drug by NHS England. It will rely on information of potentially useful treatments being flagged and sent to the relevant clinical reference group. The clinical reference group will assess those therapies for commissioning that have been assigned a promising innovative medicine designation and/or positive scientific opinion by the MHRA. It will then put in place a steering group to discuss outcome measures, cost effectiveness of the treatment, identify how many and which centres could evaluate the treatment and the need for developing additional diagnostic tests. Information on promising treatments for Early Access to Medicines Scheme commissioning will then be sent to the clinical priorities advisory group for recommendation and then to NHS England Board for final sign off. NHS England has not yet set a timeline for

Early Access to Medicines Scheme drug commissioning and it is currently unclear at what stage National Institute for Health and Care Excellence will be involved in the commissioning process.

The expectation from the MHRA is that between five and 10 drugs will go through the scheme each year.

## Clinical perspective

The NHS views the Early Access to Medicines Scheme as providing new opportunities for patients to access innovative medicines, as well as having the potential to improve the care and experience of patients where existing treatments are ineffective. It could also offer an alternative avenue for patients to enter clinical trials for drugs that are not being routinely evaluated in clinical trials in the UK. The Early Access to Medicines Scheme provides further opportunities for NHS England to work in partnership with industry to improve patient outcome and could be particularly useful in treating cancer and rare diseases. The medicines given priority for use will be those where there are no viable treatment options and where the drug is between 18 months to 2 years from obtaining a licence.

NHS England will contribute specialist professionals and advice in the form of its clinical reference groups and trust personnel, support standard diagnostic testing with the treatment and also treat adverse events. Currently, it is not clear how many NHS centres will take part in the Early Access to Medicines Scheme. The number of centres involved in the Early Access to Medicines Scheme for particular treatments will depend on the patient population for that indication and the therapies will be used in specialist centres geographically spread across the UK. It is envisaged that the Drugs and Therapeutics Committees will not intervene to prevent prescribing of therapies in the Early Access to Medicines Scheme as they would support this scheme.

The Early Access to Medicines Scheme will offer a consistent approach to measuring the value of medicines within the

NHS, as well as the provision of a controlled environment to deliver treatments in specialist centres, where clinicians will monitor and report any serious adverse events in a timely manner. In return, industry partners will need to provide the therapy free of charge up to the point of licence (the NHS will pay for patients in the Early Access to Medicines Scheme to access the drug when it is licensed) and fund any new diagnostic tests required to evaluate treatment.

The Early Access to Medicines Scheme will allow drugs to be tested in a 'real world' setting by clinicians who understand the disease and the issues around it. This will help produce data which will support both a licence application and NICE assessment, as well as provide information on patient cohorts to steer companies to apply for a licence in indications where their drug will be most effective.

### Future challenges

The Early Access to Medicines Scheme is recognized by all parties as having many benefits in providing access to unlicensed, promising treatments to patients who have life-threatening or rare disease indications. However, while the initial framework for applying for the Early Access to Medicines Scheme is now in place, issues affecting the uptake and implementation of the scheme continue to be the subject of much discussion.

Particular challenges include access to data, with industry experts and patient group representatives saying that there needs to be flexibility with the scheme so that therapeutics with solid phase II data can also enter the scheme. Many believe that for rare disease indications, phase II data should be the accepted standard rather than the exception for the scientific opinion. Funding for the scheme is another area of discussion, as there is currently no cen-

tral reimbursement of drugs made available through the Early Access to Medicines Scheme. Industry maintains that central reimbursement would improve uptake and make this scheme a more viable proposition for smaller companies in particular. Further clarity around Early Access to Medicines Scheme commissioning by NHS England as well as the process for National Institute for Health and Care Excellence evaluation and timelines will also help to resolve uncertainties and increase uptake of the scheme.

It should be noted that the MHRA's scheme is separate to, but complements the European Medicines Agency's adaptive licensing scheme (European Medicines Agency, 2014) which is designed to grant marketing authorization of drug candidates to a restricted patient group.

Early Access to Medicines Scheme is now open for business. It is anticipated that the scheme will allow UK-based patients earlier access to much needed innovative therapies, and that the data arising from the use of these experimental therapies will also contribute to increased understanding of dis-

eases, how therapies work and how the NHS can operate most effectively to deliver on patients' needs. **BJHM**

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## KEY POINTS

- The newly launched Early Access to Medicines Scheme will allow patients with rare and life-threatening or seriously debilitating conditions access to medicines that do not yet have marketing authorization.
- The Early Access to Medicines Scheme has three stages: promising innovative medicine designation, scientific opinion and commissioning.
- The scientific opinion will be used to help clinicians and patients to make a treatment decision on the use of the medicine before its licence has been approved.
- Step III – commissioning of the drug by NHS England – will involve assessment by the relevant clinical reference group and recommendation by the clinical priorities advisory group.
- The Early Access to Medicines Scheme provides further opportunities for NHS England to work in partnership with industry to improve patient outcome.
- Industry partners will need to provide the therapy free of charge up to the point of licence and fund any new diagnostic tests required to evaluate treatment.
- This scheme complements the European Medicines Agency's adaptive licensing scheme.

## CORRESPONDENCE

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